

Stem Cell Agency Board Approves Latest Steps in CIRM 2.0, Allocates \$190 million to Drive the Full Spectrum of Stem Cell Research

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Board also approves almost \$16 M to treat AIDS-lymphoma and rare immune deficiency disease

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Oakland, CA – CIRM 2.0, the California stem cell agency's plan to radically overhaul the way it works, took another big step forward today when the CIRM Board voted in favor of two new concepts covering Discovery – or early stage – and Translational research, and to renew two existing educational programs.

"When we launched the clinical phase of CIRM 2.0 on January 1 we declared this was just the start in creating a new way of funding research. Today we built on that start," says C. Randal Mills, Ph.D., President and CEO of CIRM. "The Board's approval of our proposals for Discovery - the earliest phase of research - and Translational - the phase needed to help move the most promising work towards clinical trials - helps us cover the full spectrum of stem cell research."

"What makes this approach different is that under CIRM 2.0 we are creating a pathway for research, from Discovery to Translational and Clinical, so that if they are successful they are able to move ahead into the next phase. We are not interested in research just for its own sake. To live up to our mission we want to do all that we can to make sure we accelerate the most promising research and develop therapies for patients with unmet medical needs."

In the Discovery program, for example, there are financial incentives for researchers who successfully complete their work, and then move it along into the Translational phase – either themselves or by finding a scientific partner willing to do that.

By scheduling both the Discovery and Translational awards more regularly CIRM is creating a system that helps move the most promising projects along a pipeline and also provides predictability to the grant system, making it easier for researchers to know when they can apply for funding.

The stem cell agency estimates that each year there will be up to 50 Discovery awards worth a total of \$53 million; 12 Translation awards worth a total of \$40 million; and 12 clinical awards worth around \$100 million.

"CIRM 2.0 not only helps speed up the funding process, making it easier for researchers to get the money they need when they need it, it also helps provide a tangible vision of our financial future," says Jonathan Thomas, Ph.D., J.D., Chair of the Board. "Knowing how often these programs are going to be offered, and how much money is likely to be awarded means the Board has more information to work with in making decisions on where best to allocate our funding."

The Board also agreed to invest \$15.9 million in clinical trials aimed at helping people battling AIDS-related Lymphoma and X-linked Chronic Granulomatous Disease.

Dr. Mehrdad Abedi at the University of California, Davis, was awarded \$8.5 million to treat people with HIV/AIDS who have lymphoma. The team will take the individual's own hematopoietic or blood stem cells, genetically modify them so they contain a triple combination of anti-HIV genes, and then re-introduce them to the individual. It's hoped this will help reconstitute the immune systems of AIDS-lymphoma patients.

Dr. Don Kohn and his team at the University of California, Los Angeles, were awarded \$7.4 million to run a clinical trial for people with X-linked Chronic Granulomatous Disease. This is an immune deficiency disease that leaves people unable to fight off bacterial or fungal infections, having a severe impact on both the quality of life and life expectancy. While extremely rare - there are only 20 new cases reported in the U.S. every year - the impact on those affected is substantial and the Board felt that supporting this approach could potentially help these individuals and their families and advance the field.

"The decision to invest in these therapies is just one part of the work we do to try and help them succeed," says Dr. Mills. "Our mission is to accelerate the development of therapies for patients with unmet medical needs and money is an important part of that, but so is the support we provide by assigning each clinical program a Clinical Advisory Panel."

These CAPs consist of subject matter experts, CIRM Science Officers and a Patient Advocate and work with the researchers, guiding and advising them and doing all they can to help them succeed.”

The Board also approved five more years of funding to two other CIRM 2.0 programs, Bridges and SPARK (formerly called Creativity), adding in more patient-focused activities to the existing curricula.

The Bridges program offers stem cell research training and experience to a diverse group of undergraduate and Masters level students. The goal of the program is to help support the development of the next generation of stem cell researchers, one reflecting the diversity of California.

The Summer Program to Accelerate Regenerative Medicine Knowledge, (SPARK) is designed to inspire and engage high school students and encourage them to consider a career in stem cell research. The program focuses on high school students who represent the diversity of California’s population and who might not otherwise have opportunities to take part in summer research internships due to socioeconomic constraints.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today’s most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world’s largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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